WE ACCELERATE CURES

The MMRF is dedicated to finding innovative ways to fight cancer. Our mission is to accelerate cures and save lives.

Before the MMRF, there were no new treatments in the past decade. Today, myeloma has seen more drugs approved than any other cancer.

We have built an end-to-end system in precision medicine that collects a wide range of patient data, encourages the open sharing of data, and accelerates clinical trials to make treatments available faster.

We are working in partnership with today’s leading companies and research institutions to develop molecularly targeted, immune and novel therapies, to get the right treatment to the right patient, at the right time.

Because the MMRF was founded by a patient, our system was built around the patient. It is the reason we are able to deliver better results, faster and more efficiently.

themmrf.org
Partner Highlights

The MMRF partners with the best and brightest from the academic, biotech and pharmaceutical fields. We work with leading researchers and scientists to conduct research — not fund research conducted by other companies.

“Progress is happening so fast in multiple myeloma research because the MMRF is driving everyone to work together toward a common goal. It should be happening in every cancer.”

Eric Lander, PhD
President, Founding Director
Broad Institute of MIT and Harvard

“With the MMRF, we will accelerate many of the traditional steps in the drug discovery and development process, and bypass obstacles that delay or prevent promising treatments from reaching patients.”

Colin Hill BS, MS
CEO, Chairman and Co-Founder
GNS Healthcare

“The MMRF model fuels early stage disease investigation and the development of powerful molecular and immunological tools to bring us one step closer to eliminating the disease altogether.”

William H. Hait MD, PhD
Global Head, Research and Development, Janssen
MMRF ACHIEVEMENTS
UNMATCHED RESULTS IN RECORD TIME

The MMRF has opened over 70 trials of 35 new therapies. Ten drugs have been approved by the FDA for multiple myeloma. Survival is three times longer than when we started.

70 trials  35 new treatments tested  10 drugs approved  3X lifespan

THE MMRF PRECISION MEDICINE MODEL

The Data Bank
It all starts with data, so we created ways to generate it, gather it, decode it, and track it over time. The MMRF Data Bank is a goldmine of longitudinal genomic and clinical data.

The Learning Network
By pushing our valuable data to the public domain and creating incentives for academia and industry to share their learnings (instead of safeguarding them until they are published), we accelerate research and discovery.

The Clinic
Data and learning produce treatments to be tested. Our own clinical network, the Multiple Myeloma Research Consortium, helps us speed promising new therapies to trial, and the patients who need them.
The MMRF CoMMpass Study is the cornerstone of our Precision Medicine Initiative, and is now yielding extraordinary insights into new targets for drug development as well as new ideas on how to identify and treat high-risk patients.

Launched in 2011, the MMRF CoMMpass Study (Clinical Outcomes in Multiple Myeloma to Personal Assessment of Genetic Profiles) is the first large-scale, longitudinal study in multiple myeloma. It is a $40 million effort funded by the MMRF in conjunction with our pharmaceutical partners.

In 2015, the CoMMpass Study reached its target enrollment of 1,000 multiple myeloma patients, a remarkable achievement for a relatively rare disease, and a testament to the commitment of patients to help advance the study of multiple myeloma. Baseline characteristics of the CoMMpass population show broad representation and are characteristic of myeloma patients in the general population. The average age was 64 years and the majority of participants were males of non-Hispanic/non-Latino descent. Approximately 16% of CoMMpass patients self-reported as African American, an important feature, given the higher incidence of persons of African descent in the myeloma population compared to the general population.

The MMRF CoMMpass Study involves an active assessment schedule, including bone marrow samples when first diagnosed, to establish a baseline, then again at response to treatment, and at relapse. Each patient is followed for up to eight years and their genomic and clinical data is analyzed every six months.

“The MMRF CoMMpass Study is the single most important thing going on in the multiple myeloma world.”

– David Siegel, MD, PhD
Chief of Multiple Myeloma
Hackensack University Medical Center
What Have We Learned?

Important findings have begun to emerge from the MMRF CoMMpass Study. To begin, we’ve validated that enrollees in the study proportionately match the multiple myeloma community as a whole in terms of age and ethnicity.

- Preliminary findings demonstrate improved progression-free survival with triplet therapy versus doublet therapy.
- Research also indicates improved progression-free survival with triplet therapy followed by stem cell transplant compared to triplet therapy alone.
- Integrative analyses using CoMMpass data will help identify patients at greater risk of progression at diagnosis and optimize their treatment from the beginning of their disease course.
- CoMMpass is helping to confirm and expand the list of genes that are altered and likely play a role in myeloma.

Given the preliminary nature of these findings, further work must be done to confirm the results and share them with the research community. Eventually, these data will help contribute to a more precise treatment pathway for each multiple myeloma patient based on the characteristics of his or her individual disease.

What We Expect to Learn in the Future from CoMMpass:

As the CoMMpass data continues to mature, it is our hope that the findings will help to answer some of the questions most important to patients, including:

**For Patients:**
- What treatment should I start on?
- Are there treatments specific to my myeloma subtype?
- Should I have a transplant?
- Should I be on maintenance therapy?
- If and when I relapse, what treatments will be best for me?

**For Clinicians:**
- Which patients should receive more (or less) aggressive therapy upfront?
- Should all patients receive some form of maintenance therapy?
- How should I sequence and combine therapies for individual patients?
- How do I identify high-risk patients?
- What is the prognostic value of minimal residual disease (MRD)?
In early 2016 the MMRF launched a partnership with the University of Michigan to complete clinical-grade sequencing for 500 myeloma patients to match them to precision based trials based on the results. We spoke to Dr. Ravi Vij, Principal Investigator at Washington University about the impact of this protocol.

**Dr. Ravi Vij, MD**

**Why is this initiative so important to patients?**

From the MMGI and CoMMpass, we have learned that myeloma shares some of the same genomic alterations that are observed in other cancers. In addition a significant percentage of multiple myeloma patients have at least one genomic alteration against which there is a drug currently in clinical development or even approved in other cancers which we will refer to as “actionable mutations”.

Clinical trials are being opened to treat myeloma patients with targeted agents directed at specific genomic mutations.

**How many sites are participating?**

The protocol will open at nearly all MMRC centers. Institutions who have the protocol open are now able to send patients’ samples to the University of Michigan for genomic profiling and receive sequencing information back in less than two weeks.

**What can we expect for next steps following the profiling of the patients?**

The hope is to find actionable mutations that can be used to tailor treatments with drugs that have been proven to be effective in other cancers with identical or similar mutations. The patient and the physician will review the clinical grade results together and develop a treatment plan. The rapid turn-around time is extremely important because patients who are relapsing want to be able to identify their next treatment option quickly.

**How could this study benefit participating and non-participating patients?**

Molecular and clinical de-identified data from this study will be stored in a public portal that researchers will be able to access. The data will be analyzed and used for research purposes to better understand the biology of the disease and lead to targeted drug treatment.
The Learning Network encompasses initiatives designed to share data and facilitate collaboration and discovery, such as the Gateway web portals, and drive data analytics, such as the partnership with Gene Network Sciences (GNS). Translational research initiatives, also a part of The Learning Network, help bring potential therapies from “bench to bedside” by testing theoretical discoveries generated in The Data Bank stage in a laboratory setting prior to testing human subjects.

Researcher and CoMMunity Gateway Web Portals

The Researcher Gateway was launched in 2013 as an open-access research portal serving as a data repository for CoMMpass and other genomic and/or clinical studies. Building on the MMRF’s experience with the MMGP, the Researcher Gateway is designed to have a user-friendly interface that enables clinicians and researchers to view data sets and enter queries. The web portal houses genomic, clinical, and outcomes data in one easily accessible location. Patient data are de-identified to protect patient privacy and allow researchers to extract only the specific information in which they are interested.

- Integrates clinical, laboratory, genomic data; advanced visualization, analytics
- Enables population stratification, biomarker and target discovery
- Enables researchers to connect, share analyses, results, insights

The Community Gateway enables CoMMpass Study participants and other multiple myeloma patients to connect with each other, based on their subtype and specific characteristics. The web portal also allows myeloma patients to be matched with ongoing clinical trials according to their unique profile and treatment needs.

- Over 3500 patients, patient friends and family members from 50 countries
- Special Interest Groups based on their disease characteristics
- Myeloma experts respond to questions from members

Partnership with GNS Healthcare

One notable collaboration for the MMRF is with GNS Healthcare, a leading precision medicine company that applies causal machine learning technology to match health interventions to individual patients. Using data generated by CoMMpass, the MMRF/GNS Healthcare partnership, also termed the Myeloma Disease Model, is identifying potential drivers of clinical outcomes and their associated molecular pathways.

Translational Network

The MMRF recently established and supports a transformative MMRF Translational Network of Excellence, which is focused on the most promising research on novel preclinical models for new targets and drug validation, immune biology, immune therapeutics, and minimal residual disease in myeloma and myeloma-related diseases. This groundbreaking initiative has been made possible due to decade-long efforts by the MMRF to generate assets and create a highly integrated clinical consortium.
In 2016, the MMRF had 16 active trials at year-end. Highlights include:

**A Study of Atezolizumab (Anti-Programed Death-Ligand 1 (PD-L1) Antibody) Alone or in Combination with an Immunomodulatory Drug and/or Darzalex (Daratumumab) in Participants with Multiple Myeloma.**

This nationwide trial evaluating atezolizumab (atezo) has recently expanded to include three additional arms: atezo and Darzalex (dara); atezo, dara, and Revlimid (lenalidomide); and atezo, dara, and pomalidomide. Atezo is an antibody against PD-L1 (programmed death ligand 1) and helps T-cells recognize cancer cells, so they can be killed.

**Phase 1 & 2 Trial of Idasanutlin in Combination with Ninlaro (Ixazomib) and dexamethasone in Patients with 17p Deleted, Relapsed Multiple Myeloma.**

This dose-finding trial is being conducted at sites in California, Michigan and Minnesota for patients with 17p deleted, relapsed MM to evaluate the safety of the combination of idasanutlin, an oral inhibitor of MDM2, and Ninlaro (ixzomib), a second-generation proteasome inhibitor, along with dexamethasone. Idasanutlin may work to cause cells to undergo self-destruction.

**A Phase 11 Study of IRD (Ninlaro (Ixazomib), Revlimid (Lenalidomide) and Dexamethasone) for Consolidation Therapy Post Autologous Stem Cell Transplantation Followed by Maintenance Ninlaro (Ixazomib) or Lenalidomide for Multiple Myeloma.**

This multi-site study is evaluating minimal residual disease (MRD) following autologous stem cell transplant and treatment with IRD, a three-drug regimen consisting of Ninlaro (Ixazomib), Revlimid (lenalidomide) and dexamethasone and randomization to maintenance therapy with either Ninlaro (ixzomib) or Revlimid (lenalidomide). The purpose of this trial is to study the safety and efficacy of this combination therapy in an effort to prolong the amount of time patients remain disease-free after receiving a transplant.

**MMRC Clinical Trials**

**Smoldering Multiple Myeloma (SMM):**

- The MMRC’s first trial in SMM is sponsored by Janssen Biotech, Inc. and is investigating the effects of Darzalex, an anti-CD38 antibody.

- The MMRC activated a second study in SMM, which is being led by Irene Ghobrial, MD, at the Dana-Farber Cancer Institute. Dr. Ghobrial’s interest in pursuing her study in SMM stemmed from a recently reported randomized phase III study in SMM patients in which an active drug treatment with Revlimid (lenalidomide)/dexamethasone (Rev/Dex) was compared to placebo. It is with these results that Dr. Ghobrial seeks to investigate adding a novel biological therapy, Empliciti (elotuzumab), an anti-SLAMF7 antibody, into the treatment regimen of Rev/Dex.

**Molecularly Targeted, Relapsed/Refractory with 17p deletion:**

- The MMRC has opened a Phase 1 / 2 trial, to evaluate the combination of Idasanutlin (an MDM2 inhibitor) the new oral proteasome inhibitor Ninlaro (Ixazomib), and dexamethasone in patients with 17p deletion who have relapsed. Mayo-Rochester is the lead site with Dr. Shaji Kumar as the lead PI. There are seven additional MMRC subsites participating.
MMRF 2016 Source of Funds*

- 47% Private Contributions
- 23% Healthcare Corporations
- 29% Events
- 8% Fundraising
- 3% Administrative Costs
- 1% Other

*Based on gross revenue

MMRF 2016 Spending Allocations

- 89% Research Awards and Programs
- 26% The Data Bank
- 26% The Learning Network
- 20% The Clinic
- 33% Education

8% Fundraising
3% Administrative Costs
# Multiple Myeloma Research Foundation, Inc.

## Statements of Activities (Audited) – Years Ended December 31, 2015 and December 31, 2016

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<th>Support and Revenue</th>
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| Change in net assets | ($1,824,952) | $8,808,259 |
| Net assets, beginning of year | 18,763,718 | 16,938,766 |
| Net assets, end of year    | 16,938,766  | 25,747,025  |
2016 Corporate Information

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Pat Williams
Bob Woodruff
Lee Woodruff
Multiple Myeloma Research Foundation Mission

The MMRF relentlessly pursues innovative means that accelerate the development of next-generation multiple myeloma treatments to extend the lives of patients and lead to a cure. An outstanding 90% of the MMRF total budget goes directly to research and related programming, consistently earning us top rankings from the nation's leading charity evaluators.